Scientists create 20 disease-specific stem cell lines

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(PhysOrg.com) -- Harvard Stem Cell Institute researcher George Q. Daley, associate director of the Stem Cell Program at Children's Hospital Boston, has with HSCI colleagues Chad Cowan and Konrad Hochedlinger of Massachusetts General Hospital produced a robust new collection of disease-specific stem cell lines, all of which were developed using the new induced pluripotent stem cell (iPS) technique. The work is described in a paper published in today's online edition of the journal *Cell.*

The new iPS lines, developed from the cells of patients ranging in age from one month to 57-years old and suffering from a range of conditions from Down Syndrome to Parkinson's disease, will be deposited in a new HSCI iPS Core laboratory being established at Massachusetts General Hospital (MGH), HSCI co-director Doug Melton announced yesterday. The operations of the iPS Core will be overseen by a faculty committee, which Daley will chair.

The cell lines the researchers produced carry the genes or genetic components for 10 different diseases, including Parkinson's Disease, Type I diabetes, Huntington's Disease, Down Syndrome, a form of combined immunodeficiency ("Bubble Boy's Disease"), Lesch-Nyhan syndrome, Gaucher's Disease, and two forms of Muscular Dystrophy, among others.

"We wanted to produce a large number of disease models for ourselves, our collaborators, and the stem cell research community to accelerate research," Daley said. "The original embryonic stem cell lines are generic, and allow you to ask only basic questions. But these new lines are valuable tools for attacking the root causes of disease. Our work is just the beginning for studying thousands of diseases in a petri dish," he said.

Melton, who is also co-chairman of Harvard's new interfaculty Department of Stem Cell and Regenerative Biology, said that the HSCI iPS Core will serve as a repository for iPS cells produced by HSCI scientists. The Core will also function as a technical laboratory to produce these disease-specific lines for use by scientists around the world, Melton said. He said that the cells in the iPS Core would be made available to scientists worldwide, at a nominal fee to cover costs.

Melton went on to say that "the suite of iPS cell lines reported by the Daley group marks an important achievement and a very significant advance for patients suffering from degenerative diseases. These disease-specific iPS cells are invaluable tools that will allow researchers to watch the development of diseases in petri dishes, outside of the patients. And we have good reason to believe that this will make it possible to find new treatments, and eventually drugs, to slow or even stop the course of a number of diseases. In years ahead," Melton said, "this report will be seen as opening the door to a new approach to develop therapies."

"One of our goals in creating the NIH Director's Pioneer and New Innovator Award programs was to enable exceptionally creative scientists to move quickly in promising new directions, thereby speeding the intellectual and technical breakthroughs needed to address major challenges in biomedical or behavioral research," said National Institutes of Health Director Elias A. Zerhouni, M.D. "This is certainly the case for Drs. Daley and Hochedlinger, who deployed their Director's Award resources to advance our ability to use induced pluripotent stem cells for disease-specific studies and drug development."

Daley and his colleagues, led by first-author and Children's researcher In Hyun Park, Ph.D., intentionally produced some stem cell lines for highly heritable, single-gene diseases, such as Gaucher's; complex genetic syndromes, such as Down; and then complex diseases, such as

Parkinson's, that involve genetic, cellular, and perhaps environmental components.

"The cell lines available from the iPS Core will allow stem cell researchers around the world to explore possible gene therapies for some conditions, and will aid in the development of drugs for others," Daley said.

While Daley, Immediate Past-President of the International Society for Stem Cell Research, is enthusiastic about the promise of reprogramming studies, he is far from ready to abandon experiments with embryonic stem cells. Daley believes that reprogramming and ESC research must advance in tandem to bring cell therapy to the clinic as quickly as possible.

Provided by Harvard Stem Cell Institute